
CNS10-NPC-GDNF delivered into the motor cortex for the treatment of ALS

Grant Award Details

CNS10-NPC-GDNF delivered into the motor cortex for the treatment of ALS

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-12319

Project Objective: To assess the safety, feasibility and potential efficacy of CNS10-NPC-GDNF delivered to the hand knob of the motor cortex of ALS patients in a phase 1/2a trial.
The open label trial will explore two doses in the non dominant side. If safety is observed, this will be followed by a cohort testing the high dose in the dominant side.
If successful, rescue of upper motor neurons and hand function will be observed. Potential efficacy will be explored by comparing the rate of loss of hand strength between the treated and untreated side.

Investigator:

Name:	Clive Svendsen
Institution:	Cedars-Sinai Medical Center
Type:	PI

Disease Focus: Amyotrophic Lateral Sclerosis, Neurological Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: \$11,990,372

Status: Active

Grant Application Details

Application Title: CNS10-NPC-GDNF delivered into the motor cortex for the treatment of ALS

Public Abstract:**Therapeutic Candidate or Device**

CNS10-NPC-GDNF - a neural progenitor cell secreting GDNF

Indication

Amyotrophic Lateral Sclerosis (ALS)

Therapeutic Mechanism

This therapy will replace damaged astrocytes. The new astrocytes will release paracrine factors. As the cells have been modified to release GDNF they will also provide this neuroprotective factor to dying motor neurons in the motor cortex.

Unmet Medical Need

There are currently only two FDA-approved therapies with minimal benefits, but there is no cure for ALS. Thus there is a huge unmet medical need to find additional therapies with longer-lasting benefits.

Project Objective

Phase 1/2a completed

Major Proposed Activities

- Enrollment of 16 patients to a Phase 1/2a trial to demonstrate safety and preliminary efficacy of the cellular product
- GMP manufacturing of a new CNS10-NPC-GDNF working cell lot for future clinical trials in ALS.

Statement of Benefit to California:

ALS is a devastating disease and there are over 6,000 cases in CA. If this treatment works it will provide one of the only ways to slow down motor neuron disease progression. This illness costs the state of California millions of dollars in healthcare costs and immense suffering to those Californians affected by the disease. Additionally, because over 90% of the funds will be used in California, it will help stimulate the economy.

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